

# Agenda for the Public Meeting on Patient-Focused Drug Development for Fragile X Syndrome

<b>TIME</b>	<b>DESCRIPTION</b>	<b>PRESENTER/MODERATOR</b>
10:00 – 10:05 AM	<b>Welcome</b>	<b>Linda Sorensen</b> Executive Director, NFXF
10:05 – 10:10 AM	<b>Opening Remarks</b>	<b>Randi Hagerman, MD</b> University of California, Davis MIND Institute & Founder of the National Fragile X Foundation
10:10 – 10:20 AM	<b>OVERVIEW:</b> FDA’s Patient-Focused Drug Development Initiative	<b>Bernard Fischer, MD</b> Deputy Director for the Division of Psychiatry (Office of New Drugs), FDA
10:20 – 10:30 AM	<b>OVERVIEW:</b> Fragile X Syndrome and Current Treatment Options	<b>Elizabeth Berry-Kravis, MD, PhD</b> Professor, Pediatrics, Neurological Sciences and Biochemistry, Rush University Medical Center
10:30 –10:40 AM	<b>OVERVIEW:</b> Clinical Trial Endpoints/Outcome Measures (or lack thereof)	<b>David Hessel, PhD</b> Professor, Department of Psychiatry, UC Davis
10:40 – 10:45 AM	<b>OVERVIEW:</b> Discussion Format	<b>Linda Sorensen</b> Executive Director, NFXF
10:45 – 11:15 AM	<b>TOPIC 1*</b> Health Effects and Daily Impacts of Fragile X Syndrome	<b>Marcia Braden, PhD</b> Clinical Psychologist
<i>A panel of individuals and caregivers provide comments to start the discussion.</i>		
11:15 AM – 12:15 PM	<b>TOPIC 1*</b> Large-Group Facilitated Discussion	<b>NFXF Team</b>
<i>Individuals and caregivers in the audience are invited to add to the dialogue.</i>		

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12:15 – **BREAK**  
12:30 PM

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12:30 – **TOPIC 2\*\***  
1:00 PM Current Approaches to Treatment

**Craig Erickson, MD**  
Associate Professor, Pediatrics and  
Psychiatry, Cincinnati Children's Hospital  
Medical Center

*A panel of individuals and caregivers provide comments to start the discussion.*

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1:00 – **TOPIC 2\*\***  
2:00 PM Large-Group Facilitated Discussion

**NFXF Team**

*Individuals and caregivers in the audience are invited to add to the dialogue.*

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2:00 – **Closing Remarks**  
2:05 PM

**Linda Sorensen**  
Executive Director, NFXF

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**\*Topic 1: Health Effects and Daily Impacts of Fragile X Syndrome**

1. Which 1-3 symptoms of FXS have the most significant impact? Are there specific activities or skills that are impacted?
2. How does FXS impact daily life on the best days? How about the worst days?
3. How has your/your child's FXS changed over time? Is today different from the past? Why or why not?
4. What worries you most about your/your child's FXS?

**\*\*Topic 2: Current Approaches to Treatment**

1. If you had to choose, what are the top symptoms of FXS you would most like a treatment to target?
  2. What are you doing now to treat FXS? What factors do you consider when choosing a treatment? How has your treatment regimen changed over the years? Do you consider it effective?
  3. What are the biggest gaps in you/your child's current treatment(s)? What are the biggest downsides?
  4. What specific things would you look for in an ideal treatment for you/your child's FXS? Think about how much of a change/effect you would need to see to use a new treatment assuming there may be mild-moderate side effects.
    - a. What impact level would convince you to trial a treatment? For example, a treatment that positively affects what % of patients with FXS.
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